

Charter of the TREAT-NMD Alliance

Established (1st January 2012); Amended (25th January 2016)

Document History

Charter document first drafted in August 2011. Charter reviewed by TREAT-NMD partners, Task Force and STAC members in November 2011. Revised version sent to TREAT-NMD partners, Task Force and STAC members in December 2011 for second review. Revised charter reviewed by TREAT-NMD Alliance Executive Committee in March 2012. Charter revised to incorporate membership information, reviewed and request to approve by the TREAT-NMD Alliance Executive Committee in July 2012. Charter approved on 6th August 2012.

Charter amended and approved by the Executive Committee on 2nd October 2014.

Charter amended to reflect majority vote by Executive Committee to allow Executive Committee members to be employees of a pharmaceutical company or a not-for-profit venture philanthropy pharmaceutical company. Those members would be precluded from holding (Vice)-Chair positions on the Executive Committee. Charter approved by the Executive Committee on 25th January 2016.

Background

The mission of TREAT-NMD is to provide the neuromuscular field with the infrastructure needed to ensure that the most promising new therapies reach patients as quickly as possible. Since its launch in January 2007 the network's focus has been on the development of tools that industry, clinicians and scientists need to bring novel therapeutic approaches through preclinical development and into the clinic, and on establishing best-practice care for neuromuscular patients worldwide.

Following negotiations with the European Union (EU) by EURORDIS, AFM and academics, TREAT-NMD was initially established as an international network set up under EU funding (contract number 036825) building on preliminary work done by patient organisations to develop and optimize the tools and resources that accelerate therapy development and delivery for inherited neuromuscular diseases. The initial network encompassed over 350 researchers and clinicians in 22 European organisations, and the network expanded to include a large number of collaborators not only across Europe, but around the world.

In 2010, the major outcome from a Public Consultation was the endorsement that TREAT-NMD should continue to deliver and develop its platform of tools and resources for the neuromuscular field. This includes:

- helping industry and researchers identify the centres that have capacity and experience to conduct trials via the **care and trial site registry** (CTSR)
- utilising our international **patient registry** platform to identify the patients to recruit into them, in accordance with study inclusion criteria
- providing access to **biobanks** containing patient samples,
- providing advice (and consensus) on appropriate **standard operating procedures** and **animal models**
- facilitating the selection of appropriate validated **outcome measures**
- offer guidance on overall development programmes via **TREAT-NMD Advisory Committee for Therapeutics** (TACT)
- facilitating the development, updating, translation and dissemination of **standards of care** and monitor implementation of **standards of care**

- organising and hosting **international meetings and conferences** to update and educate the field on key issues
- working towards the discovery of **biomarkers**
- engaging with the **regulatory authorities**, representing the neuromuscular field on key issues
- debating **social and ethical issues** of importance to the field

Although the 5 years of EU funding for TREAT-NMD, ended in December 2011, thanks to support received and detailed below, we are in a position to continue to work with the neuromuscular community to help deliver promising therapies to the community quicker, for the foreseeable future. Many of the core, and platform-specific activities listed above, of the TREAT-NMD Alliance are currently supported by the following organizations:

- **Patient Organisations supporting platform activities:** Fondazione Telethon, Swiss Foundation for Muscle Disease, Parent Project Muscular Dystrophy, Cure Duchenne, Foundation to Eradicate Duchenne, Jain Foundation, Deutsche Gesellschaft für Muskelkranke e.V. (DGM), Limb Girdle Muscular Dystrophy 2I Research Fund, Association Française contre les Myopathies (AFM), Muscular Dystrophy Campaign (MDC), Jennifer Trust for SMA, Myotonic Dystrophy Support Group
- **Academic Institutions supporting core activities:** Children's National Medical Centre, Newcastle University, INSERM, University of Freiburg
- **European Commission FP7 Grants:** RD-Connect, Neuromics, COMET, Rare-best practise, Optimistic
- **National funding to support patient registries and educational meetings in many countries**

Further information regarding support for the TREAT-NMD Alliance can be found on the web site at www.treat-nmd.eu.

TREAT-NMD has been highlighted by many as an example of a successful network that has fostered successful collaborations around the world, and should continue this facilitation role for the community. To achieve this, the resources developed by TREAT-NMD will be further developed by the TREAT-NMD Alliance and coordinated by many of those who have helped to build the TREAT-NMD platform as a well-recognised brand.

Input in to the composition and governance of the TREAT-NMD Alliance has been developed through consultation with the TREAT-NMD EU Network partners as well as consultation with academic leaders and patient organisations.

The academic members of the newly established TREAT-NMD Alliance Executive Committee (www.treat-nmd.eu/executive-committee) were nominated by the Task Force. The Patient Organisation representatives of the Executive Committee were nominated and elected by the Patient community, via a consultation involving approximately 120 organisations. Members of the Executive Committee were elected for an initial term of 2 years. The Executive Committee is supported by a Secretariat based at Newcastle University.

Article I – Organisation and Affiliation

- 1.1 This organisation is known as the TREAT-NMD Alliance. It is a platform of tools and resources to advance diagnosis, care and treatment for those living with neuromuscular disorders around the world. Since its launch in January 2007 with EU funding the mission of TREAT-NMD has been to provide tools and resources that will accelerate translational research for neuromuscular diseases and bring new therapies and treatments to patients in the most efficient and effective means possible.
- 1.2 The TREAT-NMD Alliance is organised as an independent, non-profit consortium of interested parties who work together to support therapy delivery in neuromuscular diseases. The views of the TREAT-NMD Alliance do not necessarily represent the views of the institutions and organisations with which members are affiliated.

Article II – Membership

- 2.1 A Member of the TREAT-NMD Alliance agrees to sign a membership form and agrees to work with the Alliance to further advance the aims and objectives of the Alliance.
- 2.2 To apply for membership a completed membership form should be signed and submitted to the Secretariat of the Alliance. Membership forms will be approved by the Executive Committee.
- 2.3 On becoming a member, members will receive a membership certificate and will be listed as a member on the TREAT-NMD website (name and location), unless requested otherwise. Members representing organisations may be required to provide their logo for the TREAT-NMD website.
- 2.4 Members who wish to terminate their membership should contact the Secretariat of the Alliance in writing at any time. Membership will cease after 30 days from receiving the termination request and the members' information will be removed from the web site within this period.
- 2.5 Members will have access to reports, including financial reports, newsletters, work plans and updates on activities.
- 2.6 Members must not, whether knowingly or negligently, act in a way which could bring the Alliance into disrepute or lead to a possible loss of public confidence.
- 2.7 If a member is found to have brought, or could possibly bring, the Alliance into disrepute then the member will be contacted by the Chair of the Executive Committee in writing to request an explanation of the reasons for their actions and how this can be resolved. The member will be given a period of 30 days to resolve the issue(s) or explain why they feel they have not contravened the obligations in article 2.6. If the member does not comply then they will be asked to remove the TREAT-NMD logo and any other association from any publishable materials, i.e., websites, newsletters, etc and their membership will be terminated forthwith following a two thirds vote of the Executive Committee members.
- 2.8 Members will not claim to be a member of the Alliance without first having signed the membership form referred to in article 2.2.
- 2.9 Members must comply with all relevant known national and international data protection, privacy and ethical laws and guidelines.

Article III – Membership Meetings

- 3.1 Membership meeting: The date of the membership meeting will be set by the Executive Committee who will also set the time and the place. This will be announced at least six months in advance.
- 3.2 Special meetings: Special meetings (either in person or virtual) may be called by the Executive Committee.

3.3 Notice: Notice of meeting agendas and supporting documents for each meeting will be given to each member, by (electronic) mail, not less than fourteen (14) days before the meeting.

Article IV – Executive Committee

- 4.1 The Executive Committee is responsible for providing overall policy and strategic direction to the TREAT-NMD Alliance, oversees activities and progress, and delegates responsibility for day-to-day operations to the representatives of the various Activity Groups. The Executive Committee will have no more than 13 and not fewer than 8 members. The Executive Committee receives no compensation other than reasonable expenses. Members of the Executive Committee are elected as private individuals and not representatives of their home institution or organisation.
- 4.2 Only members can be elected into the Executive Committee and four of the Executive Committee will be elected from individuals nominated by patient organisation or advocacy groups. The remaining nine of the Executive Committee membership will be composed of academic members of the TREAT-NMD Alliance. Individuals nominated to be elected into the Executive Committee may be employees of a pharmaceutical company or a not-for-profit venture philanthropy pharmaceutical company, but are precluded from holding (Vice)-Chair positions on the Executive Committee.
- 4.3 The Executive Committee will meet at least annually, either in person or virtually, at an agreed upon time and place.
- 4.4 Election of new Executive Committee members or election of current Executive Committee members to a second term will occur as the first item of business at the annual meeting. Executive Committee members will be elected by a ranked vote of the current TREAT-NMD Alliance members. A separate ballot will be held for the patient organisation candidates and the academic candidates, as required.
- 4.5 All Executive Committee members will serve 2 year terms, but are eligible for re-election, with at least three Executive Committee positions up for rotation every 2 years.
- 4.6 A quorum must be attended by at least two thirds of the Executive Committee members before business can be transacted or motions made or passed.
- 4.7 An Executive Committee meeting requires that each Executive Committee member have written notice of the agenda at least two weeks in advance.
- 4.8 There will be two officers of the Executive Committee consisting of a Chair and Vice-Chair. Their duties are as follows:
 - 4.8.1 The Chair will convene regularly scheduled Executive Committee meetings, will preside or arrange for the Vice-Chair or other members of the Executive Committee to preside at each meeting. The Chair will serve a 1 year term of office. This term can be extended for an additional year by a two thirds vote of the Executive Committee members.
 - 4.8.2 The Vice-Chair will assume the role of Chair following a 1 year term of office, and will chair subcommittees on special subjects as designated by the Executive Committee, unless the current Chair is re-elected for an additional 1 year term of office as described in article 4.8.1, the Vice-Chair will serve a 2 year term of office.
- 4.9 When a vacancy on the Executive Committee exists, nominations for new members may be received from present TREAT-NMD Alliance members by the Secretariat two weeks in advance of an Executive Committee meeting. These nominations will be sent out to the members to be voted upon and results announced at the next Executive Committee meeting. These vacancies will be filled only to the end of the particular Executive Committee member's term. A separate ballot will be held for the patient organisation candidates and the academic candidates, as required.
- 4.10 Resignation from the Executive Committee must be in writing and received by the Secretariat. An Executive Committee member will be removed from the Executive Committee if s/he has excess absences from regular Executive Committee meetings following a three-quarter vote of the remaining Executive Committee members following an appeal. An Executive

Committee member may be removed for other reasons by a three-fourths vote of the remaining Executive Committee members following an appeal. All appeals must be submitted to the Chair of the Executive Committee on request.

Article V – Secretariat to the Executive Committee

- 5.1 The Secretariat to the Executive Committee will be provided by Newcastle University, until such a time when this role is transferred to another member organisation who can take over the administrative and financial responsibility for this role and whose role is agreed by a two thirds vote of the Executive Committee.
- 5.2 The Chair of the Executive Committee will be provided administrative support to ensure efficient communication between the Chair, other members of the Executive Committee, the membership and the Secretariat.
- 5.3 The Secretariat will provide an ongoing advisory role to the Executive Committee and members, as well as administrative support, communications support (web site, newsletter), and project management support where required and is dependent on sufficient funding available to support these roles.
- 5.4 The Secretariat is responsible for supporting the activities of the Executive Committee, coordinating the implementation of the strategic priorities, fiscal procedures, support the development of donations/fundraising and grant plans and providing annual activity and financial reports to the TREAT-NMD Alliance members. The Executive Committee must approve the annual reports.
- 5.5 The Secretariat will provide all supporting documentation and information to the Executive Committee at their regular meetings.
- 5.6 The Secretariat will be responsible for keeping records of Executive Committee actions, including overseeing the taking of minutes at all Executive Committee meetings, sending out meeting announcements, distributing copies of minutes and the agenda to the Executive Committee and members, and assuring that records are maintained. The Secretariat will also provide an annual office report to the Executive Committee describing the activities and financial status of the Secretariat.
- 5.7 The fiscal year will be the calendar year. Annual reports will be submitted and reported to the Executive Committee showing income, expenditures, justifications, and pending income. The financial records of the TREAT-NMD Alliance are public information and will be made available to the membership, Executive Committee members and the public.
- 5.8 The Secretariat will also be responsible for planning and delivering all meetings (virtual and in person) of the Executive Committee, as well as any other meetings deemed necessary by the Executive Committee.

Article VI – Subcommittees and Advisory Boards

- 6.1 The Executive Committee may create subcommittees and advisory boards as needed, such as fundraising, membership, conference, etc.
- 6.2 The Executive Committee Chair appoints all subcommittee and advisory board chairs upon agreement by the Executive Committee. All subcommittees and advisory boards will report to the Executive Committee.
- 6.3 Advisory Boards will be required to report and provide recommendations to the Executive Committee based on the remit of the advisory board as defined by the Executive Committee. These bodies include, but not limited to:

6.4 TREAT-NMD Global Databases Oversight Committee

- 6.4.1 The TREAT-NMD Global Databases Oversight Committee (TGDOC) is the governing structure of the TREAT-NMD global databases on behalf of associated national and international TREAT-NMD registries according to the registry charter.

- 6.4.2 The TGDOC is composed of representatives of patient organizations, and registry representatives and some experts. Currently, a third of members are patients or patient representatives.
- 6.4.3 The TGDOC has a current chair, chair-elect and past chair; with the chair being elected annually (amended charter). Industry shall not be represented in the committee.
- 6.4.4 All members of the TGDOC must disclose financial interests and update the disclosure statements on an annual basis. All members of the TGDOC will be required to sign confidentiality agreements if a third party requests them prior to having access to the inquiry of the third party.
- 6.4.5 The TGDOC will meet in person at least once per year, and by teleconference or by e-communication.
- 6.4.6 The TGDOC will report to the TREAT-NMD Alliance Executive Committee and to the TREAT-NMD registries annually.
- 6.4.7 The TGDOC reviews inquiries of third parties into the TREAT-NMD global databases. The TGDOC will come to a decision within 14 calendar days upon receipt of the inquiry and will report the decision in writing to the third party and the owner of the global database as per the approval process agreed by the TGDOC. If a decision cannot be reached, the inquiry shall be rejected. In the case of a rejection, the TGDOC may report the reason for rejection to the third party and set a time frame for reconsideration.

6.5 TREAT-NMD Advisory Committee for Therapeutics

- 6.5.1 The TREAT-NMD Advisory Committee for Therapeutics (TACT), is an expert multidisciplinary body that provides the neuromuscular community (clinicians, researchers, patient advocacy groups and industry) with independent and objective guidance on advancing new therapies (whether novel or repurposed) for neuromuscular diseases.
- 6.5.2 Its goal is to position the potential therapy along a realistic pathway to eventual clinical trial and registration by evaluating preclinical data as well as drug development considerations that are crucial for the conduct of studies that generate meaningful data.
- 6.5.3 The remit of the committee is to provide applicants with transparent and consistent guidance and advice, in an educational and directional context, on the readiness of drugs and/or therapeutic targets for trial. TACT advice will be helpful for preparing funding applications and investigational drug applications, while the publication of a non-confidential summary will ensure the TREAT-NMD Alliance and wider community receives expert feedback on the progress of the application.
- 6.5.4 TACT reviews also address issues of drug formulation, bioavailability and toxicology as well as possible regulatory requirements and marketing considerations.
- 6.5.5 TACT is also dedicated to providing information on rational and economical trial design. The ultimate goal of TACT is to help pave the pathway for successful drug registration by providing professional and independent advice.
- 6.5.6 The TACT committee convenes once every six months to consider selected applications. TACT will consider more than one drug at a time and therefore will have panels of experts, selected from the TACT members, reviewing the drugs concurrently, with the same chair and core committee for continuity.

Article VII – TREAT-NMD Alliance Publication Policies

- 7.1 All publications involving work by members that was supported by the TREAT-NMD Alliance (financially or in kind) should be acknowledged by the main author as described in article 7.2 of this charter and a copy of the publication should be forwarded to the Secretariat. Members are encouraged to use open source journals for publication whenever possible.
- 7.2 The TREAT-NMD Alliance should be duly acknowledged in the acknowledgements section of the manuscript with the following text: “This work has been supported by the TREAT-NMD Alliance”. If a journal policy complies and the Alliance contribution is deemed significant in accordance

with publication good practice, it is recommended that the author line conclude with the statement “in conjunction with the TREAT-NMD Alliance*”. The asterisk (*) refers to the TREAT-NMD web site address as a footnote on the first page of the manuscript (www.treat-nmd.eu).

Article VIII – Conflict of Interest Guidelines

- 8.1 The TREAT-NMD Alliance wishes to promote independence, objectivity and a fair balance of representation, in all its activities. Members of the Executive Committee, of the secretariat and of advisory boards and subcommittees (where applicable), are expected to disclose their financial or in-kind relationships with their organisations, industry and any other organisation that could represent a potential Conflict of Interest. Full disclosure is expected even when it is not clear whether a relationship or affiliation constitutes a conflicting interest.
- 8.2 The TREAT-NMD Alliance recognises that these relationships do not necessarily imply bias or decrease the value of participation in professional activities.
- 8.3 Disclosure of these relationships is necessary for others to make an informed decision about the impact of the disclosed relationship. All relationships over the previous two calendar years and the current year (including future commitments which are foreseen over the coming year) must be disclosed. Executive Committee members, secretariat members and advisory board and subcommittee members are required to renew their disclosures on an annual basis.
- 8.4 Members of the TREAT-NMD Alliance should maintain the highest personal and professional standards in conducting activities. TREAT-NMD Alliance members have an obligation to act in the public interest and in the interests of the TREAT-NMD Alliance, and should be willing to educate the scientific and lay communities.

Article IX – Date of Activation of Bylaws and Interim Officers

- 9.1 When approved by at least two-thirds of the votes cast by the Executive Committee, these bylaws will become active and adopted at the time of the business meeting.

Article X – Amendment of Charter Statement

- 10.1 These bylaws may be amended when necessary by a two-thirds majority of the Executive Committee. A quorum of at least three-fourths is required. Proposed amendments must be submitted to the Secretariat to be sent out with regular Executive Committee announcements.

Article XI – Dissolution of the TREAT-NMD Alliance

- 11.1 The dissolution of the TREAT-NMD Alliance will be decided by an affirmative vote by written ballot of three-fourths of votes of the members of the TREAT-NMD Alliance. The procedure on dissolution will follow that outlined for proposing amendments in Article X.
- 11.2 If the TREAT-NMD Alliance is wound up, all of the Alliance’s assets which would otherwise be available to its members generally shall be transferred on its winding up either to another body with objects similar to that of the Alliance or to another body the objects of which are the promotion of charity and anything incidental or conducive thereto (whether or not the body is a member of the Alliance).

These revised Bylaws were approved following a meeting and vote of the Executive Committee of the TREAT-NMD Alliance on 25th January 2016.